Krystal Biotech Announces Publication of Phase 1 and 2 Clinical Trial (GEM 1/2 Study) of Beremagene Geperpavec (B-VEC) Data in Nature Medicine

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- *Treatment with B-VEC demonstrated robust functional COL7 expression followed by its assembly into basement membrane-associated anchoring fibrils*
- *Treatment with B-VEC improved durable wound closure in patients with recessive dystrophic epidermolysis bullosa (RDEB) compared with placebo with minimal adverse events*

PITTSBURGH, March 28, 2022 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc., ("Krystal") (NASDAQ: KRYS), the leader in redosable gene therapies for rare diseases, today announced that results from the Phase 1 and 2 study of topical beremagene geperpavec (B-VEC) for the treatment of dystrophic epidermolysis bullosa (DEB) were published in *Nature Medicine*.

The publication provides a comprehensive analysis of the data from the Phase 1 and 2 study showing that repeat topical applications of B-VEC were associated with durable wound closure, full-length cutaneous type VII collagen (COL7) expression, and anchoring fibril assembly with minimal reported adverse events.

"In this first-ever clinical trial of a redosable topical gene therapy, we are pleased to see that these data show the potential of B-VEC to address the underlying cause of the disease and delineate B-VEC as an easily administered, well tolerated therapy," said Suma Krishnan, President, Research and Development, Krystal Biotech, Inc. "For so many years, all we have been able to offer DEB patients was palliative care, so it is now gratifying to have a potential corrective treatment option for this deserving group of patients. We are grateful to the trial participants who made this study possible."

The study was led by senior author M. Peter Marinkovich, M.D., Director of the Blistering Disease Clinic at Stanford Health Care and Associate Professor of Dermatology at the Stanford University School of Medicine.

DEB is a rare and severe disease that affects the skin and mucosal tissues. It is caused by one or more mutations in the *COL7A1* gene, which is responsible for the production of the protein COL7 that forms anchoring fibrils that bind the dermis (inner layer of the skin) to the epidermis (outer layer of the skin). The lack of functional anchoring fibrils in DEB patients leads to extremely fragile skin that blisters and tears from minor friction or trauma. DEB patients suffer from open wounds, leading to skin infections, fibrosis that can cause fusion of fingers and toes, and ultimately an increased risk of developing an aggressive form of squamous cell carcinoma that, in severe cases, can be fatal.

In the Phase 1 and 2 study, matched wounds were evaluated in nine RDEB patients receiving topical B-VEC or placebo repeatedly over 12 weeks. Primary and secondary mechanistic and clinical endpoints were met. No Grade 2 or above B-VEC-related adverse events, vector shedding, or systemic drug exposure were noted.

About B-VEC
B-VEC is an investigational non-invasive, topical, redosable gene therapy designed to deliver two copies of the *COL7A1* gene when applied directly to DEB wounds. B-VEC was designed to treat DEB at the molecular level by providing the patient's skin cells the template to make normal COL7 protein, thereby addressing the fundamental disease-causing mechanism.

The FDA and EMA have each granted B-VEC orphan drug designation for the treatment of DEB, and the FDA has granted B-VEC fast track designation and rare pediatric designation for the treatment of DEB. In addition, in 2019, the FDA granted Regenerative Medicine Advanced Therapy (RMAT) to B-VEC for the treatment of DEB and the EMA granted PRIority MEdicines ("PRIME") eligibility for B-VEC to treat DEB.

About Krystal Biotech
Krystal Biotech, Inc. (NASDAQ:KRYS) is a pivotal-stage gene therapy company leveraging its proprietary, redosable gene therapy platform and in-house manufacturing capabilities to
potentially bring life-changing treatment options to patients with serious diseases, including rare diseases in skin, lung, and other areas. For more information, please visit http://www.krystalbio.com, and follow @KrystalBiotech on LinkedIn and Twitter.

Forward-Looking Statements
Any statements in this press release about future expectations, plans and prospects for Krystal Biotech, Inc., including statements about the clinical utility of B-VEC and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "likely," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: uncertainties associated with regulatory review of clinical trials and applications for marketing approvals, the availability or commercial potential of product candidates including B-VEC, the sufficiency of cash resources and need for additional financing and such other important factors as are set forth under the caption "Risk Factors" in Krystal's annual and quarterly reports on file with the U.S. Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent Krystal's views as of the date of this release. Krystal anticipates that subsequent events and developments will cause its views to change. However, while Krystal may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing Krystal's views as of any date subsequent to the date of this release.

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